August 24th 2009

Dear Friends,

We are writing to you, at what is a very difficult and worrying time for the global Gaucher community, to provide an update on the supply of Cerezyme and how the matter is being dealt with across Europe.

As you will no doubt be aware, on the 13th/14th June, a virus was found at the Genzyme facility in the United States used for the production of Cerezyme. Although there is no evidence that the virus affects humans, the plant was shut down and production was halted whilst it was sanitised. Production has restarted and it is anticipated that the first supplies of the new product will commence in mid November with full supplies of Cerezyme being available from the beginning of next year.

It was initially anticipated that the impact of the shutting of the plant would be to limit supplies of therapy (globally) to 60% of those previously supplied. Based on this assumption the EU stakeholders group met and issued guidelines which we circulated in our last email. However the figure of 60% of supply assumed that all the Cerezyme that was in the course of production could be used for patients , but it became apparent that in order to use much of this material it would have been necessary to reintroduce it into the sanitised plant. Genzyme decided not to do this for fear that the material may be contaminated, which would have resulted in the facility having again to be closed and resanitised. The effect of this decision is that 80% of the Cerezyme in production could not be used after all.

On the 10th August Genzyme decided that if supplies were to be available to the most needy patients that the global supply would need to be cut to approximately 20% of the previous normal dose until the end of the year. This of course has very serious and immediate consequences for all patients.

Attached is a slide presentation prepared by Genzyme setting out the sequence of events which explains the situation in detail.

We also attach a letter and press release from the European Medicines Agency (EMEA) which offers guidelines on the allocation of the Cerezyme which remains available. You will see that they recommend that priority is given to children, neuronopathic patients and adult patients with severe life threatening progressive disease. This does mean that all patients receiving treatment will see a dose reduction and many patients will not receive any treatment at all for some months. This is clearly not desirable but is unavoidable. This supply management plan assumes that two additional lots will be released by EMEA/FDA. These two lots were finished but not released before the facility used for the production of Cerezyme was shut down. Genzyme is currently in discussions with EMEA/FDA regarding their release. We do expect EMEA to inform about this at the end of this month and will inform you by mail as soon as we know more.

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We do know that plans are being developed in many countries on a national basis. The board of the EGA have met and we have agreed that we wish to see consistency across Europe in the implementation of the EMEA recommendations and in dosage levels as well as in the management of this crisis. We have raised this with Genzyme and have been in discussions with European doctors who we know are very keen to work together to ensure that the most vulnerable patients receive priority and to shape and define priorities on a pan European basis.

With the doctors we have also been working to explore the availability of alternative therapies. As you will know Zavesca (Miglustat) is an oral therapy which is approved for use in mild to moderate Gaucher disease. There are possible side effects but the EMEA do make reference to Zavesca in their guidelines and some doctors are considering this for their patients.

You will also be aware of the ongoing (and fairly advanced) clinical trials for two new enzyme therapies (Velaglucerase from Shire and prGCD produced by Protalix). Neither therapies yet have a product licence but both have received special approvals from the US Food and Drug Administration (FDA) for their trail protocols for use. As yet, we do not know whether either company has any availability of product, and we are seeking further information on whether either of these trial drugs could be offered to patients. We attach also copies of press releases issued by Shire and Protalix which refer to the FDA approval given to both companies for the protocols they have presented for the use of their products.

In addition Genzyme are running trials of their "small molecule" therapy (GENZ-112638) which may be considered further.

We have also been in touch with the National Gaucher foundation in the United States and they are very keen to work with us in seeking to address what is truly a global challenge.

In order to be most effective we do need to share information. Therefore please tell us how this situation is being handled in your countries. We want to be able to feed back to the companies and share your experiences with the doctors. This will help them shape policies and reactions to the current situation. If there is anything that you think that we can do to help you please do tell us.

So please mail back to tell us what is happening. It really is important that we are able to share accurate information and that we can build a picture of the impact of this very serious situation across Europe.

Finally we want to thank the doctors in all the countries who we know are working very hard to try to ensure that that most needy patients are prioritised. They are having to take very difficult decisions which affect our members and of course we appreciate all their efforts.

We will be in touch with you again as further information becomes available. In the meantime we look forward to hearing from you

With good wishes

Jeremy Manuel O.B.E.
Chairman European Gaucher Alliance

Anne Grethe Lauridsen
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